

DEC 2004

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property Organization
International Bureau(43) International Publication Date
24 December 2003 (24.12.2003)

PCT

(10) International Publication Number
WO 03/106645 A2

(51) International Patent Classification⁷: **C12N**
 (21) International Application Number: **PCT/US03/18923**
 (22) International Filing Date: 17 June 2003 (17.06.2003)
 (25) Filing Language: English
 (26) Publication Language: English
 (30) Priority Data:
 10/174,014 17 June 2002 (17.06.2002) **US**

(71) Applicant (for all designated States except US): **ISIS PHARMACEUTICALS, INC.** [US/US]; 2292 Faraday Avenue, Carlsbad, CA 92008 (US).

(72) Inventors; and

(75) Inventors/Applicants (for US only): **BENNETT, Clarence, Frank** [US/US]; 1347 Cassins Street, Carlsbad, CA 92009 (US). **FREIER, Susan, M.** [US/US]; 2946 Renault Street, San Diego, CA 92122 (US). **DOBIE, Kenneth, W.** [GB/US]; 703 Stratford Court, #4, Del Mar, CA 92014 (US).

(74) Agents: **CALDWELL, John, W.** et al.; Woodcock Washburn LLP, One Liberty Place - 46th Floor, Philadelphia, PA 19103 (US).

(81) Designated States (*national*): AE, AG, AL, AM, AT, AU, AZ, BA, BB, BG, BR, BY, BZ, CA, CIL, CN, CO, CR, CU, CZ, DE, DK, DM, DZ, EC, EE, ES, FI, GB, GD, GH, GM, HR, HU, ID, IL, IN, IS, JP, KE, KG, KP, KR, KZ, LC, LK, LR, LS, LT, LU, LV, MA, MD, MG, MK, MN, MW, MX, MZ, NO, NZ, OM, PH, PL, PT, RO, RU, SC, SD, SE, SG, SK, SL, TJ, TM, TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, YU, ZA, ZM, ZW.

(84) Designated States (*regional*): ARIPO patent (GH, GM, KE, LS, MW, MZ, SD, SL, SZ, TZ, UG, ZM, ZW), Eurasian patent (AM, AZ, BY, KG, KZ, MD, RU, TJ, TM), European patent (AT, BE, BG, CH, CY, CZ, DE, DK, EE, ES, FI, FR, GB, GR, HU, IE, IT, LU, MC, NL, PT, RO, SE, SI, SK, TR), OAPI patent (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW, ML, MR, NE, SN, TD, TG).

Published:

— without international search report and to be republished upon receipt of that report

For two-letter codes and other abbreviations, refer to the "Guidance Notes on Codes and Abbreviations" appearing at the beginning of each regular issue of the PCT Gazette.

(54) Title: ANTISENSE MODULATION OF SMRT EXPRESSION

(57) **Abstract:** Antisense compounds compositions and methods are provided for modulating the expression of SMRT. The compositions comprise antisense compounds, particularly antisense oligonucleotides, targeted to nucleic acids encoding SMRT. Methods of using these compounds for modulation of SMRT expression and for treatment of diseases associated with expression of SMRT are provided.

WO 03/106645 A2